



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

14 December 2021
EMA/PDCO/695889/2021
Human Medicines Division

Paediatric Committee (PDCO)

Draft Agenda for the meeting on 14-17 December 2021

Chair: Koenraad Norga – Vice-Chair: Sabine Scherer

14 December 2021, 11:00- 19:00, Virtual meeting

15 December 2021, 08:30- 19:00, Virtual meeting

16 December 2021, 08:30- 19:00, Virtual meeting

17 December 2021, 08:30- 13:00, Virtual meeting

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the PDCO Committee meeting reports (after the PDCO Opinion is adopted), and on the Opinions and decisions on paediatric investigation plans webpage (after the EMA Decision is issued).

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).



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1. Introductions

1.1. Welcome and declarations of interest of members, alternates and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the PDCO plenary session to be held 14-17 December 2021. See December 2021 PDCO minutes (to be published post January 2022 PDCO meeting).

1.2. Adoption of agenda

PDCO agenda for 14-17 December 2021

1.3. Adoption of the minutes

PDCO minutes for 9-12 November 2021

2. Opinions

Some information related to this section cannot be disclosed at the present time as it is deemed to contain commercially confidential information.

2.1. Opinions on Products

2.1.1. EMEA-002958-PIP01-21

Treatment of hypertrophic cardiomyopathy

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

2.1.2. Seralutinib - Orphan - EMEA-002972-PIP01-21

Gossamer Bio 002 Limited; Treatment of pulmonary arterial hypertension

Day 120 opinion

Action: For adoption

Cardiovascular Diseases

Note: Withdrawal request received on 24 November 2021

2.1.3. Single strain of non-genetically modified *Prevotella histicola* - EMEA-002933-PIP01-20

Treatment of psoriasis

Day 120 opinion

Action: For adoption

Dermatology

2.1.4. EMEA-002992-PIP01-21

Treatment of fibrodysplasia ossificans progressiva (FOP)

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.5. Ethinylestradiol / dienogest - EMEA-002229-PIP02-21

Treatment of polycystic ovary syndrome

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.6. Poly(oxy-1,2-ethanediyl), alpha-hydro-omega-methoxy, ether with N-[[[2-[[6-[[1-[3-[[3-(2,3-dihydroxypropoxy)propyl]amino]-3-oxopropyl]-2,5-dioxo-3-pyrrolidinyl]thio]hexyl]amino]ethyl]amino]carbonyl]-2-methylalanyl-teriparatide (2:1) - Orphan - EMEA-002955-PIP01-21

Ascendis Pharma Bone Diseases A/S; Treatment of hypoparathyroidism

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.7. Tildacerfont - Orphan - EMEA-002970-PIP01-21

Spruce Biosciences, Inc.; Treatment of congenital adrenal hyperplasia

Day 120 opinion

Action: For adoption

Endocrinology-Gynaecology-Fertility-Metabolism

2.1.8. Benralizumab - EMEA-001214-PIP07-21

Treatment of eosinophilic gastritis/eosinophilic gastroenteritis

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.9. Izencitinib - EMEA-002757-PIP02-21

Treatment of Crohn's disease

Day 120 opinion

Action: For adoption

Gastroenterology-Hepatology

2.1.10. 6-[(3S,4S)-4-methyl-1-(pyrimidin-2-ylmethyl)pyrrolidin-3-yl]-3-tetrahydropyran-4-yl-7H-imidazo[1,5-a]pyrazin-8-one - Orphan - EMEA-002991-PIP01-21

IMARA Inc; Treatment of sickle cell disease

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

Note: Withdrawal request received on 10 December 2021

2.1.11. Benralizumab - EMEA-001214-PIP04-19

Treatment of hypereosinophilic syndrome (HES)

Day 120 opinion

Action: For adoption

Haematology-Hemostaseology

2.1.12. Alectinib - EMEA-002431-PIP02-21

Treatment of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 120 opinion

Action: For adoption

Oncology

2.1.13. Nirogacestat hydrobromide - Orphan - EMEA-002971-PIP01-21

SpringWorks Therapeutics, Inc; Treatment of soft tissue sarcoma

Day 120 opinion

Action: For adoption

Oncology

[2.1.14. Ribociclib - EMEA-002765-PIP02-21](#)

Treatment of neuroblastoma

Day 120 opinion

Action: For adoption

Oncology

[2.1.15. Vorasidenib - EMEA-002932-PIP02-21](#)

Treatment of glioma

Day 120 opinion

Action: For adoption

Oncology

[2.1.16. EMEA-003002-PIP01-21](#)

Treatment of proteinuric chronic kidney disease

Day 120 opinion

Action: For adoption

Uro-nephrology

[2.1.17. ExPEC9V - EMEA-002996-PIP01-21](#)

Prevention of *E.coli* infections / Prevention of infections caused by extraintestinal pathogenic *Escherichia coli* (ExPEC)

Day 120 opinion

Action: For adoption

Vaccines

[2.1.18. Acetylsalicylic acid / rosuvastatin calcium - EMEA-002239-PIP02-21](#)

Prevention of cardiovascular events

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.19. Colchicine - EMEA-003101-PIP01-21

Reduction of atherothrombotic events in patients with coronary artery disease

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.20. Derivative of pyrrolopyrimidine - EMEA-003109-PIP01-21

Prevention of cardiovascular outcome events in patients with heart failure

Day 60 opinion

Action: For adoption

Cardiovascular Diseases

2.1.21. Fostamatinib - EMEA-001196-PIP03-21

Treatment of autoimmune haemolytic anaemia

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation / Haematology-Hemostaseology

2.1.22. Gantenerumab - EMEA-003107-PIP01-21

Alzheimer's disease

Day 60 opinion

Action: For adoption

Neurology

2.1.23. Aumolertinib - EMEA-003106-PIP01-21

Treatment of lung cancer

Day 60 opinion

Action: For adoption

Oncology

2.1.24. Pembrolizumab / favezelimab - EMEA-003104-PIP01-21

Treatment of malignant neoplasms of the central nervous system / Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue)

Day 60 opinion

Action: For adoption

Oncology

2.2. Opinions on Compliance Check

2.2.1. Tralokinumab - EMEA-C2-001900-PIP02-17-M05

LEO Pharma A/S; Treatment of atopic dermatitis

Day 60 letter

Action: For adoption

Dermatology

2.2.2. Simeticone / macrogol 4000 / potassium chloride / sodium sulphate, anhydrous / sodium chloride / citric acid, anhydrous / sodium citrate - EMEA-C-001356-PIP02-12-M04

Alfasigma S.p.A.; Bowel cleansing prior to clinical procedures

Day 60 opinion

Action: For adoption

Gastroenterology-Hepatology

2.2.3. Peramivir - EMEA-C-001856-PIP02-16-M02

BioCryst Ireland Limited; Treatment of influenza

Day 60 opinion

Action: For adoption

Infectious Diseases

2.2.4. Pneumococcal polysaccharide serotype 1 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 3 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 4 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 5 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 6A – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 6B – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 7F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 9V – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 14 – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 18C – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 19A – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 19F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 22F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 23F – diphtheria CRM197 conjugate / pneumococcal polysaccharide serotype 33F – diphtheria CRM197 conjugate (15-

valent pneumococcal polysaccharide conjugate vaccine [V114]) - EMEA-C-002215-PIP01-17-M03

Merck Sharp & Dohme (Europe), Inc.; Prevention of disease caused by *Streptococcus pneumoniae*

Day 60 opinion

Action: For adoption

Vaccines

2.3. Opinions on Modification of an Agreed Paediatric Investigation Plan

2.3.1. Dupilumab - EMEA-001501-PIP02-13-M07

Sanofi-aventis groupe; Treatment of asthma

Day 60 opinion

Action: For adoption

Dermatology

2.3.2. Mitapivat - Orphan - EMEA-002684-PIP01-19-M01

Agios Netherlands B.V.; Treatment of pyruvate kinase deficiency

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.3. Vadadustat - EMEA-001944-PIP01-16-M03

Otsuka Pharmaceutical Development & Commercialisation Europe GmbH; Treatment of anaemia due to chronic disorders

Day 60 opinion

Action: For adoption

Haematology-Hemostaseology

2.3.4. Allogeneic bone marrow derived mesenchymal stromal cells, ex vivo expanded - Orphan - EMEA-002706-PIP01-19-M01

Medac Gesellschaft für klinische Spezialpräparate mbH; Treatment of acute graft-versus-host disease

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.5. Apremilast - EMEA-000715-PIP03-11-M07

Amgen Europe B.V.; Treatment of psoriasis

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

Note: Withdrawal request received on 8 December 2021

2.3.6. Baricitinib - EMEA-001220-PIP01-11-M06

Eli Lilly and Company Limited; Treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis)

Day 60 opinion

Action: For adoption

Immunology-Rheumatology-Transplantation

2.3.7. Avibactam / ceftazidime - EMEA-001313-PIP01-12-M11

Pfizer Europe MA EEIG; Treatment of bacterial infections

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.8. Cabotegravir - EMEA-001418-PIP01-13-M04

ViiV Healthcare UK Limited; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.9. Cobicistat / atazanavir sulphate - EMEA-001465-PIP01-13-M04

Bristol-Myers Squibb Pharma EEIG; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.10. Tenofovir alafenamide / rilpivirine / emtricitabine - EMEA-001679-PIP01-14-M01

Gilead Sciences International Ltd.; Treatment of human immunodeficiency virus (HIV-1) infection

Day 60 opinion

Action: For adoption

Infectious Diseases

2.3.11. Brivaracetam - Orphan - EMEA-000332-PIP02-17-M03

UCB Pharma S.A.; Treatment of neonatal seizures / Treatment of paediatric epilepsy syndromes

Day 60 opinion

Action: For adoption

Neurology

2.3.12. Onasemnogene abeparvovec - Orphan - EMEA-002168-PIP01-17-M04

Novartis Gene Therapy EU Limited; Treatment of spinal muscular atrophy

Day 60 opinion

Action: For adoption

Neurology

2.3.13. Phenobarbital - EMEA-002532-PIP01-18-M02

Proveca Pharma Limited; Treatment of epilepsy

Day 60 opinion

Action: For adoption

Neurology

2.3.14. Avapritinib - Orphan - EMEA-002358-PIP02-18-M02

Blueprint Medicines (Netherlands) B.V.; Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Day 60 opinion

Action: For adoption

Oncology

2.3.15. Gemtuzumab ozogamicin - Orphan - EMEA-001733-PIP02-15-M02

Pfizer Europe MA EEIG; Treatment of acute myeloid leukaemia

Day 60 opinion

Action: For adoption

Oncology

2.3.16. Talimogene laherparepvec - EMEA-001251-PIP01-11-M05

Amgen Europe B.V.; Treatment of solid malignant non-CNS tumours

Day 60 opinion

Action: For adoption

Oncology

2.3.17. Cysteamine (hydrochloride) - Orphan - EMEA-000322-PIP01-08-M06

Recordati Rare Diseases SARL; Treatment of corneal cystine crystal deposits in cystinosis

Day 60 opinion

Action: For adoption

Ophthalmology

2.3.18. Lanadelumab - Orphan - EMEA-001864-PIP01-15-M06

Takeda Pharmaceuticals International AG Ireland Branch; Hereditary angioedema

Day 60 opinion

Action: For adoption

Other

2.3.19. Budesonide / glycopyrronium bromide / formoterol fumarate dihydrate - EMEA-002063-PIP01-16-M01

AstraZeneca AB; Treatment of asthma

Day 60 opinion

Action: For adoption

Pneumology - Allergology

2.3.20. Dexmedetomidine (hydrochloride) - EMEA-002758-PIP01-19-M01

BioXcel Therapeutics, Inc.; Treatment of bipolar disorder / Treatment of schizophrenia

Day 60 opinion

Action: For adoption

Psychiatry

2.4. Opinions on Re-examinations

2.4.1. Glycopyrronium bromide - EMEA-002383-PIP01-18-M01

Dr. August Wolff GmbH & Co. KG - Arzneimittel; Treatment of hyperhidrosis

Day 30 opinion

Action: For adoption

Dermatology

2.4.2. Ravulizumab - EMEA-001943-PIP04-20

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorders

Day 30 opinion

Action: For adoption

Neurology

2.5. Opinions on Review of Granted Waivers

No item

2.6. Finalisation and adoption of Opinions

No item

2.7. Partial Compliance Checks completed by EMA

The following partial compliance checks have been identified by the PME coordinator and PDCO rapporteur as not needing to be referred to the PDCO for discussion. The PDCO has been informed in writing.

2.7.1. Cotadutide - EMEA-C1-002712-PIP01-19-M01

AstraZeneca AB; Treatment of non-alcoholic steatohepatitis (NASH)

Day 30 letter

Action: For information

Immunology-Rheumatology-Transplantation

3. Discussion of applications

Some information related to this section cannot be disclosed at the present time as it is deemed to contain commercially confidential information.

3.1. Discussions on Products D90-D60-D30

3.1.1. Phospholipid esters from herring roe - EMEA-003053-PIP01-21

Treatment of psoriasis

Day 90 discussion

Action: For discussion

Dermatology

3.1.2. Efgartigimod alfa - Orphan - EMEA-002597-PIP04-21

Argenx BV; Treatment of immune thrombocytopenia

Day 90 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.3. Recombinant humanized anti-blood dendritic cell antigen 2 (BDCA2) monoclonal antibody - EMEA-002555-PIP02-21

Treatment of lupus erythematosus

Day 90 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.4. EMEA-003081-PIP01-21

Prevention of coronavirus disease 2019 (COVID-19) / Treatment of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Action: For discussion

Infectious Diseases

3.1.5. Acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) - Orphan - EMEA-002796-PIP01-20

IntraBio Ltd.; Treatment of Niemann-Pick disease type C

Day 90 discussion

Action: For discussion

Neurology

3.1.6. Firazorexton sesquihydrate - EMEA-002993-PIP01-21

Treatment of narcolepsy

Day 90 discussion

Action: For discussion

Neurology

3.1.7. Givinostat - Orphan - EMEA-000551-PIP04-21

Italfarmaco S.p.A.; Treatment of Duchenne muscular dystrophy

Day 90 discussion

Action: For discussion

Neurology

3.1.8. Viltolarsen - Orphan - EMEA-002853-PIP01-20

NS Pharma, Inc.; Treatment of Duchenne muscular dystrophy

Day 90 discussion

Action: For discussion

Neurology

3.1.9. Pemigatinib - Orphan - EMEA-002370-PIP02-21

Incyte Biosciences Distribution B.V.; Treatment of myeloid/lymphoid neoplasms with FGFR1 rearrangement

Day 90 discussion

Action: For discussion

Oncology

3.1.10. Zamtocabtagene autoleucel - Orphan - EMEA-003009-PIP01-21

Miltenyi Biomedicine GmbH; Treatment of mature B cell neoplasms

Day 90 discussion

Action: For discussion

Oncology

3.1.11. Pabinafusp alfa - Orphan - EMEA-003033-PIP01-21

JCR Pharmaceuticals Co., Ltd.; Mucopolysaccharidosis type II

Day 90 discussion

Action: For discussion

Other

3.1.12. EMEA-003052-PIP01-21

Treatment of cystic fibrosis

Day 90 discussion

Action: For discussion

Pneumology - Allergology

3.1.13. EMEA-002946-PIP01-20

Treatment of major depressive disorder

Day 90 discussion

Action: For discussion

Psychiatry

3.1.14. Ralmitaront - EMEA-003003-PIP01-21

Treatment of schizophrenia

Day 90 discussion

Action: For discussion

Psychiatry

3.1.15. L-Carnitine / glucose / calcium chloride dihydrate / magnesium chloride hexahydrate / sodium lactate / sodium chloride - EMEA-003049-PIP01-21

Treatment of patients in need of peritoneal dialysis

Day 90 discussion

Action: For discussion

Uro-nephrology

3.1.16. COVID-19 vaccine (recombinant, adjuvanted) - EMEA-002987-PIP01-21

Prevention of coronavirus disease 2019 (COVID-19)

Day 90 discussion

Action: For discussion

Vaccines

3.1.17. *Neisseria meningitidis* serogroup B fHbp subfamily B / *Neisseria meningitidis* serogroup B fHbp subfamily A / *Neisseria meningitidis* group Y polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group W-135 polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group C polysaccharide conjugated to tetanus toxoid carrier protein / *Neisseria meningitidis* group A polysaccharide conjugated to tetanus toxoid carrier protein - EMEA-002814-PIP02-21

Invasive disease caused by *Neisseria meningitidis* group A, B, C, W and Y from 2 months of age

Day 90 discussion

Action: For discussion

Vaccines

3.1.18. Insulin efsitora alfa - EMEA-003105-PIP01-21

Treatment of type 1 diabetes mellitus / Treatment of type 2 diabetes mellitus

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.19. Pudexacianinium - EMEA-003099-PIP01-21

Ureter visualisation

Day 60 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism / Diagnostic / Oncology / Gastroenterology-Hepatology / Uro-nephrology

3.1.20. Efruxifermin - EMEA-003114-PIP01-21

Treatment of non-alcoholic fatty liver disease including non-alcoholic steatohepatitis

Day 60 discussion

Action: For discussion

Gastroenterology-Hepatology

3.1.21. Omfiloctocog alfa - EMEA-003113-PIP01-21

Control and prevention of bleeding / Perioperative management

Day 60 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.22. Cenerimod - EMEA-003108-PIP01-21

Treatment of systemic lupus erythematosus (SLE)

Day 60 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.23. Adeno-associated virus serotype hu68 containing the human GLB1 gene - Orphan - EMEA-003102-PIP01-21

Passage Bio, Inc.; Treatment of GM1 gangliosidosis

Day 60 discussion

Action: For discussion

Neurology

3.1.24. Corticotropin - EMEA-003097-PIP01-21

Treatment of infantile spasms

Day 60 discussion

Action: For discussion

Neurology

3.1.25. Humanised monoclonal IgG1-based antibody - EMEA-003100-PIP01-21

Treatment of spinal muscular atrophy

Day 60 discussion

Action: For discussion

Neurology

3.1.26. Amifampridine - EMEA-003103-PIP01-21

Lambert-Eaton myasthenic syndrome

Day 60 discussion

Action: For discussion

Other

3.1.27. [2'-O-\(2'-methoxyethyl\) modified antisense oligonucleotide targeting prekallikrein \(PKK\) mRNA - EMEA-003112-PIP01-21](#)

Prevention of hereditary angioedema / Hereditary angioedema

Day 60 discussion

Action: For discussion

Pneumology - Allergology / Haematology-Hemostaseology

3.1.28. [Troriluzole - EMEA-003084-PIP02-21](#)

Treatment of obsessive-compulsive disorder

Day 60 discussion

Action: For discussion

Psychiatry

3.1.29. [EMEA-003098-PIP01-21](#)

Treatment of proteinuric chronic kidney disease

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.30. [Bardoxolone - EMEA-002488-PIP02-21](#)

Treatment of autosomal dominant polycystic kidney disease (ADPKD)

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.31. [Pegcetacoplan - Orphan - EMEA-002600-PIP03-21](#)

Apellis Ireland Limited; Treatment of glomerulonephritis and nephrotic syndrome

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.32. [Vibegron - EMEA-001415-PIP02-21](#)

Treatment of myoneurogenic bladder disorders

Day 60 discussion

Action: For discussion

Uro-nephrology

3.1.33. [Respiratory syncytial virus stabilised prefusion F subunit vaccine \(RSVpreF\) - EMEA-002795-PIP02-21](#)

Prevention of RSV-associated lower respiratory tract illness

Day 60 discussion

Action: For discussion

Vaccines

3.1.34. [EMEA-003120-PIP01-21](#)

Chronic heart failure / Prevention of cardiovascular events in patients with chronic heart failure

Day 30 discussion

Action: For discussion

Cardiovascular Diseases

3.1.35. [Ex vivo expanded autologous human keratinocytes containing epidermal stem cells transduced with a LAMB3-encoding retroviral vector - Orphan - EMEA-003137-PIP01-21](#)

Holostem Terapie Avanzate s.r.l.; Treatment of junctional epidermolysis bullosa (JEB)

Day 30 discussion

Action: For discussion

Dermatology

3.1.36. [Tezepelumab - EMEA-001613-PIP04-21](#)

Treatment of chronic spontaneous urticaria

Day 30 discussion

Action: For discussion

Dermatology

3.1.37. [Avexitide - Orphan - EMEA-003125-PIP01-21](#)

EigerBio Europe Limited; Treatment of postbariatric hypoglycaemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.38. [Elamipretide - Orphan - EMEA-003128-PIP01-21](#)

Stealth BioTherapeutics Inc.; Treatment of Barth syndrome

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.39. [Nonreplicating, recombinant adeno-associated virus \(AAV\) serotype 9 \(AAV9\) gene transfer vector that contains a modified human ATP7B coding sequence - Orphan - EMEA-003131-PIP01-21](#)

Ultragenyx Germany GmbH; Treatment of Wilson disease

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.1.40. [Parsaclisib \(as hydrochloride\) - Orphan - EMEA-002696-PIP02-21](#)

Incyte Biosciences Distribution B.V.; Treatment of autoimmune haemolytic anaemia

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.1.41. [Human normal immunoglobulin - EMEA-003121-PIP01-21](#)

Treatment of post-polio syndrome

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.1.42. [EMEA-003116-PIP01-21](#)

Celiac disease

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation / Gastroenterology-Hepatology

3.1.43. Ibrexafungerp citrate - EMEA-002535-PIP04-21

Invasive candidiasis

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.44. Nifurtimox - EMEA-003134-PIP01-21

Treatment of Chagas disease

Day 30 discussion

Action: For discussion

Infectious Diseases

3.1.45. Ivermectin - EMEA-003136-PIP01-21

Topical treatment of head lice infestations / Treatment of head lice infestations

Day 30 discussion

Action: For discussion

Infectious Diseases / Dermatology

3.1.46. Self-complementary adeno-associated viral vector serotype 9 containing the human CLN3 gene - Orphan - EMEA-003124-PIP01-21

Amicus Therapeutics Europe Limited; Neuronal ceroid lipofuscinosis - CLN3

Day 30 discussion

Action: For discussion

Neurology

3.1.47. EMEA-003129-PIP01-21

Osteosarcoma

Day 30 discussion

Action: For discussion

Oncology

3.1.48. Ceralasertib - EMEA-003127-PIP01-21

Treatment of lung carcinoma (small cell and non-small cell carcinoma)

Day 30 discussion

Action: For discussion

Oncology

3.1.49. [Cosibelimab - EMEA-003041-PIP01-21](#)

Treatment of cutaneous squamous cell carcinoma

Day 30 discussion

Action: For discussion

Oncology

3.1.50. [Nemtabrutinib - EMEA-003135-PIP01-21](#)

Treatment of mature B cell malignancies

Day 30 discussion

Action: For discussion

Oncology

3.1.51. [Sunvozertinib - EMEA-003132-PIP01-21](#)

Treatment of non-small cell lung cancer (NSCLC)

Day 30 discussion

Action: For discussion

Oncology

3.1.52. [Tarlataamab - EMEA-003138-PIP01-21](#)

Treatment of neuroendocrine prostate cancer (NEPC) / Treatment of small cell lung cancer (SCLC)

Day 30 discussion

Action: For discussion

Oncology

3.1.53. [Tucatinib - EMEA-002242-PIP02-21](#)

Treatment of malignant neoplasms with HER2 alterations / Treatment of solid tumours

Day 30 discussion

Action: For discussion

Oncology

3.1.54. Vimseltinib - Orphan - EMEA-002802-PIP02-21

Deciphera Pharmaceuticals; Treatment of tenosynovial giant cell tumour

Day 30 discussion

Action: For discussion

Oncology

3.1.55. Bemcentinib - EMEA-003123-PIP01-21

Treatment of acute myeloid leukaemia

Day 30 discussion

Action: For discussion

Oncology / Haematology-Hemostaseology

3.1.56. (R)-tetrahydrofuran-3-yl 4-(6-(5-(4-ethoxy-1-isopropylpiperidin-4-yl)pyridin-2-yl)pyrrolo[1,2-b]pyridazin-4-yl)piperazine-1-carboxylate sesquisuccinate - Orphan - EMEA-003133-PIP01-21

Ipsen Pharma; Treatment of fibrodysplasia ossificans progressiva / Fibrodysplasia ossificans progressiva

Day 30 discussion

Action: For discussion

Other

3.1.57. Anti TL1a mAb - EMEA-003111-PIP02-21

Treatment of asthma

Day 30 discussion

Action: For discussion

Other

3.1.58. Azelastine hydrochloride / mometasone furoate - EMEA-003122-PIP01-21

Seasonal allergic rhinitis

Day 30 discussion

Action: For discussion

Oto-rhino-laryngology

3.1.59. EMEA-001649-PIP02-21

Treatment of fibrosing interstitial lung diseases (ILD)

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.1.60. [Zuranolone - EMEA-003119-PIP01-21](#)

Treatment of postpartum depression

Day 30 discussion

Action: For discussion

Psychiatry

3.1.61. [Borrelia outer surface protein A \(OspA\) serotypes \(ST1-6\) lipidated, fusion protein vaccine - EMEA-003130-PIP01-21](#)

Prevention of Lyme disease

Day 30 discussion

Action: For discussion

Vaccines

3.1.62. [Recombinant COVID-19 subunit nanoparticle - EMEA-003115-PIP01-21](#)

Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Vaccines

3.2. **Discussions on Compliance Check**

The following compliance checks have been identified for discussion and the members of the PDCO have been invited to comment on issues of possible non-compliance.

3.2.1. [Remimazolam \(as besylate\) - EMEA-C1-001880-PIP02-19-M03](#)

PAION Deutschland GmbH; Sedation

Day 30 discussion

Action: For discussion

Anaesthesiology

3.2.2. [Nirsevimab - EMEA-C2-001784-PIP01-15-M03](#)

AstraZeneca AB; Prevention of lower respiratory tract infection caused by respiratory

syncytial virus

Day 30 discussion

Action: For discussion

Infectious Diseases

3.2.3. Nivolumab - EMEA-C-001407-PIP01-12-M03

Bristol-Myers Squibb Pharma EEIG; Treatment of all conditions included in the category of malignant neoplasms (except nervous system, haematopoietic and lymphoid tissue)

Day 30 discussion

Action: For discussion

Oncology

3.2.4. *Neisseria meningitidis* serogroup B recombinant lipoprotein(rLP2086;subfamily B) / *Neisseria meningitidis* serogroup B recombinant lipoprotein (rLP2086; subfamily B; *Escherichia coli*) / *Neisseria meningitidis* serogroup B recombinant lipoprotein (rLP2086; subfamily A; *Escherichia coli*) - EMEA-C3-001037-PIP02-11-M05

Pfizer Europe MA EEIG; Prevention of invasive meningococcal disease caused by *N. meningitidis* serogroup B

Day 30 discussion

Action: For discussion

Vaccines

3.3. Discussions on Modification of an Agreed Paediatric Investigation Plan

3.3.1. Delgocitinib - EMEA-002329-PIP02-20-M01

LEO Pharma A/S; Treatment of chronic hand eczema

Day 30 discussion

Action: For discussion

Dermatology

3.3.2. Spesolimab - EMEA-002475-PIP02-19-M02

Boehringer Ingelheim International GmbH; Treatment of generalised pustular psoriasis / Prevention of generalised pustular psoriasis

Day 30 discussion

Action: For discussion

Dermatology

3.3.3. Tralokinumab - EMEA-001900-PIP02-17-M06

LEO Pharma A/S; Treatment of atopic dermatitis

Day 30 discussion

Action: For discussion

Dermatology

3.3.4. Ethinylestradiol / dienogest - EMEA-002229-PIP01-17-M03

Chemo Research; Prevention of pregnancy

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.5. Pegvaliase - Orphan - EMEA-001951-PIP01-16-M02

BioMarin International Limited; Treatment of hyperphenylalaninaemia

Day 30 discussion

Action: For discussion

Endocrinology-Gynaecology-Fertility-Metabolism

3.3.6. Etrasimod L-arginine - EMEA-002713-PIP01-19-M01

Arena Pharmaceuticals, Inc.; Treatment of ulcerative colitis

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.7. Guselkumab - EMEA-001523-PIP05-19-M01

Janssen-Cilag International N.V.; Treatment of Crohn's disease

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.8. Mirikizumab - EMEA-002208-PIP01-17-M02

Eli Lilly and Company; Treatment of ulcerative colitis / Treatment of Crohn's disease

Day 30 discussion

Action: For discussion

Gastroenterology-Hepatology

3.3.9. Efanesoctocog alfa - Orphan - EMEA-002501-PIP01-18-M02

Bioverativ Therapeutics, Inc., a Sanofi Company; Treatment of haemophilia A

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.10. Glutamine - Orphan - EMEA-001996-PIP02-16-M01

Emmaus Medical Europe Ltd.; Treatment of sickle cell disease

Day 30 discussion

Action: For discussion

Haematology-Hemostaseology

3.3.11. Apremilast - EMEA-000715-PIP02-11-M05

Amgen Europe B.V.; Treatment of juvenile psoriatic arthritis (JPsA) / Treatment of juvenile idiopathic arthritis (JIA)

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.12. Imlifidase - Orphan - EMEA-002183-PIP01-17-M01

Hansa Biopharma AB; Desensitisation treatment of highly sensitised kidney transplant patients with positive crossmatch against an available donor

Day 30 discussion

Action: For discussion

Immunology-Rheumatology-Transplantation

3.3.13. Casirivimab - EMEA-002964-PIP01-21-M01

Regeneron Ireland DAC; Treatment of coronavirus disease 2019 (COVID-19) / Prevention of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.14. Cilgavimab - EMEA-002925-PIP01-20-M01

AstraZeneca AB; Prevention or treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.15. Fostemsavir (tromethamine) - EMEA-001687-PIP01-14-M06

ViiV Healthcare UK Ltd; Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.16. Imdevimab - EMEA-002965-PIP01-21-M01

Regeneron Ireland DAC; Prevention of coronavirus disease 2019 (COVID-19) / Treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.17. Ridinilazole hydrate - EMEA-002250-PIP02-17-M01

Summit Limited; Treatment of *Clostridium difficile* infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.18. Tedizolid phosphate - EMEA-001379-PIP01-12-M06

Merck Sharp & Dohme (Europe), Inc.; Treatment of acute bacterial skin and skin structure infections

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.19. Tenofovir disoproxil - EMEA-000533-PIP01-08-M11

Gilead Sciences International Limited; Treatment of chronic viral hepatitis B / Treatment of human immunodeficiency virus (HIV-1) infection

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.20. Tixagevimab - EMEA-002900-PIP01-20-M01

AstraZeneca AB; Prevention or treatment of coronavirus disease 2019 (COVID-19)

Day 30 discussion

Action: For discussion

Infectious Diseases

3.3.21. Efinaconazole - EMEA-001627-PIP01-14-M01

Almirall, S.A.; Onychomycosis (tinea unguium)

Day 30 discussion

Action: For discussion

Infectious Diseases / Dermatology

3.3.22. Isoflurane - EMEA-002320-PIP01-17-M02

Sedana Medical AB; Sedation of mechanically ventilated patients

Day 30 discussion

Action: For discussion

Neonatology - Paediatric Intensive Care

3.3.23. Delandistrogene moxeparvovec - Orphan - EMEA-002677-PIP01-19-M01

Roche Registration GmbH; Duchenne muscular dystrophy

Day 30 discussion

Action: For discussion

Neurology

3.3.24. Eculizumab - Orphan - EMEA-000876-PIP05-15-M05

Alexion Europe SAS; Myasthenia gravis

Day 30 discussion

Action: For discussion

Neurology

3.3.25. Eptinezumab - EMEA-002243-PIP01-17-M02

H. Lundbeck A/S; Prevention of migraine headaches

Day 30 discussion

Action: For discussion

Neurology

3.3.26. Dinutuximab beta - Orphan - EMEA-001314-PIP01-12-M01

EUSA Pharma (Netherlands) BV; Neuroblastoma

Day 30 discussion

Action: For discussion

Oncology

3.3.27. Ex vivo expanded autologous human corneal epithelium cells containing stem cells - Orphan - EMEA-001082-PIP02-11-M03

Holostem Terapie Avanzate S.r.l.; Limbal stem cell deficiency due to ocular burns

Day 30 discussion

Action: For discussion

Ophthalmology

3.3.28. Vosoritide - Orphan - EMEA-002033-PIP01-16-M02

BioMarin International Limited; Treatment of achondroplasia

Day 30 discussion

Action: For discussion

Other

3.3.29. Adrenaline (epinephrine) - EMEA-002749-PIP01-19-M01

ARS Pharmaceuticals IRL, Limited; Treatment of allergic reactions

Day 30 discussion

Action: For discussion

Pneumology - Allergology

3.3.30. Modified allergen extract of birch pollen - EMEA-000932-PIP01-10-M02

ROXALL Medizin GmbH; Treatment of allergic rhinitis / rhino-conjunctivitis

Day 30 discussion

Action: For discussion

- 3.3.31. Split influenza virus, inactivated containing antigens equivalent to the B-like strain (Yamagata lineage) / Split influenza virus, inactivated containing antigens equivalent to the B-like strain (Victoria lineage) / Split influenza virus, inactivated containing antigens equivalent to the A/H3N2-like strain / Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain - EMEA-002359-PIP01-18-M04
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Sanofi Pasteur; Prevention of influenza infection

Day 30 discussion

Action: For discussion

Vaccines

4. Nominations

Information related to this section cannot be disclosed at the present time as it is deemed to contain commercially confidential information.

4.1. List of submissions of applications with start of procedure 4 January 2022 for Nomination of Rapporteur and Peer reviewer

Action: For adoption

4.2. Nomination of Rapporteur for requests of confirmation on the applicability of the EMA decision on class waiver

Action: For adoption

4.3. Nominations for other activities

Action: For adoption

5. Scientific Advice Working Party (SAWP) and Paediatric Committee (PDCO) Interaction

Information related to this section cannot be disclosed at the present time as it is deemed to contain commercially confidential information.

6. Discussion on the applicability of class waivers

Some information related to this section cannot be disclosed at the present time as it is deemed to contain commercially confidential information.

6.1. Discussions on the applicability of class waiver for products

6.1.1. Lutetium (¹⁷⁷Lu) edotreotide - EMEA-15-2021

ITM Solucin GmbH; The classes of androgen receptor modulator, of oestrogen receptor modulator, of growth and sex hormone as well as their releasing or inhibiting factors, and of sex hormone-metabolism modulators medicinal products for treatment of breast malignant neoplasms, prostate malignant neoplasms and neuroendocrine malignant neoplasms / Treatment of gastroenteropancreatic neuroendocrine tumours (GEP-NETs)

Action: For adoption

7. Discussion on the inclusion of an indication within a condition in an agreed PIP/waiver

7.1. Discussion on the possibility to include an indication within a condition in an agreed PIP/waiver

No item

8. Annual reports on deferrals

Note: The annual reports on deferrals to be noted by the members of the PDCO are flagged in the Annex B.

9. Organisational, regulatory and methodological matters

9.1. Mandate and organisation of the PDCO

9.1.1. PDCO membership

Action: For information

9.1.2. Vote by proxy

Action: For information

9.1.3. Strategic Review and Learning Meeting (SRLM) – Paris, 31 March – 1 April 2022

PDCO member: Sylvie Benchetrit

Action: For information

9.2. Coordination with EMA Scientific Committees or CMDh-v

9.2.1. Committee for Medicinal Products for Human Use (CHMP)

Action: For information

9.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

9.3.1. Non-clinical Working Group: D30 Products identified

PDCO member: Karen van Malderen

Action: For information

9.3.2. Formulation Working Group - Workplan – January 2022 – December 2024

PDCO member: Brian Aylward

Action: For information

9.3.3. Formulation Working Group – List of experts for 2022

PDCO member: Brian Aylward

Action: For adoption

9.4. Cooperation within the EU regulatory network

9.4.1. European Network of Paediatric Research (Enpr) - European Medicines Agency (EMA)

Pan-European paediatric clinical trial network – Conect4children

Action: For information

9.5. Cooperation with International Regulators

No item

9.6. Contacts of the PDCO with external parties and interaction with the Interested Parties to the Committee

No item

9.7. PDCO work plan

9.7.1. Development of Therapeutic Areas strategies - update

PDCO member: Sylvie Benchetrit

Action: For information

9.8. Planning and reporting

9.8.1. EMA Business Pipeline activity and Horizon scanning

Q4/2021 Update of the Business Pipeline report for the human scientific committees

Action: For information

10. Any other business

10.1. COVID-19 update

Action: For information

10.2. Extrapolation project – presentation of results and conclusions

Action: For information

10.3. Lifecycle regulatory submissions metadata (LRSM)

Action: For information

10.4. PIP-related CHMP procedures – discussion on monitoring – call for volunteers

PDCO member: Sabine Scherer

Action: For information

11. Breakout sessions

11.1. Internal PDCO Operations

Action: For discussion on Tuesday, 11:00 - 12:00

11.2. Neonatology

Action: For discussion on Tuesday, 13:00 - 14:00

11.3. Paediatric oncology

Action: For discussion on Wednesday, 13:00 - 14:00

11.4. Vaccines

Action: For discussion on Thursday, 13:00 - 14:00

12. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

Paediatric investigation plan (PIP) (section 2.1 Opinion on PIPs and section 3.1 Discussions on PIPs)

A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, when it is safe to do so, to support the authorisation of a medicine for children. Pharmaceutical companies submit proposals for PIPs to the European Medicines Agency's Paediatric Committee (PDCO). This Committee is responsible for agreeing or refusing the plan.

Compliance checks (section 2.2 Opinions on Compliance check, section 3.2 Discussions on Compliance check)

A compliance check may be necessary before any application for marketing authorisation (even for an adult indication) can be considered valid, if there was no deferral for at least one of the studies agreed in the PIP, or after the due date of initiation or completion of a study/measure. The same applies to some regulatory applications for authorised products, as described above.

Modification of an Agreed Paediatric Investigation Plan (section 2.3 Opinions on Modification of an agreed PIP, section 3.3 Discussions on Modification of an agreed PIP)

The development plan for a medicine can be modified at a later stage as knowledge increases. Modifications can also be made if the applicant encounters such difficulties with the implementation of a PIP, which render it unworkable or no longer appropriate.

In some cases, studies can be deferred until after the studies in adults have been conducted. This ensures that research in children is done only when it is safe and ethical to do so. Even when studies are deferred, the PIP will include details of the paediatric studies and their timelines.

Class waiver (section 6 Discussion on the applicability of class waiver)

As some diseases do not affect children (for example Parkinson's disease), the development of medicines for these diseases should not be performed in children. In these cases, a PIP is not required and it will be waived. For more information on the classes of diseases subject to waivers, see [class waivers](#).

Annual reports on deferrals (section 8)

If the medicinal product is approved in the EU, annual reports on the deferred measures in the PIP must be submitted to the Agency.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/